## Research Targeting Diabetes, Liver Failure and HIV Get Support from California Stem Cell Agency

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**December 14, 2017 Oakland, CA** The California Institute for Regenerative Medicine (CIRM), the state's Stem Cell Agency, today invested almost $24.8 million dollars in 13 different projects targeting a wide range of diseases and disorders.

The investment is part of CIRM's Discovery Quest Award Program. Quest promotes the discovery of promising new stem cell-based technologies that could be translated to enable broad use and ultimately, improve patient care.

"One of the most important things CIRM is able to do is invest in early stage projects, long before other industry or private investors show any interest in them," says Maria Millan, M.D., President & CEO of CIRM. "Without our support, many of these early stage projects might never get off the ground. With our support, they may help produce something that has a profound impact on patients' lives."

San Diego-based company ViaCyte was awarded $1.47 million to develop a universal donor cell (UDC) line by genetically editing an embryonic stem cell line. They first hope to use this line to produce pancreatic cells, the kind destroyed in type 1 diabetes. If they are able to produce pancreatic cells that deliver insulin, without being attacked by a patient's immune system, they could help millions of people suffering from type 1 diabetes. If the UDC cells work for this condition they could potentially then be adapted to other diseases.

Researchers at Stanford were awarded $2.2 million to help people battling liver failure. Currently the only treatment for liver failure is a liver transplant, but there is a drastic shortage of available organs in the US and, according to the Organ Procurement and Transplantation Network (OPTN) around eight people die every day waiting for a liver transplant. The Stanford team hope to develop a method of generating large numbers of human liver cells that could be used to repair liver damage and help patients avoid the need for a transplant.

Two other projects, both of which received earlier funding from CIRM, were also successful. Neurona Therapeutics was awarded $1.6 million to develop a stem cell therapy for epilepsy. A team at UC San Diego received $2.1 million to use neural stem cells to help repair the damage caused by a spinal cord injury. These awards reflect the Stem Cell Agency's efforts to create a pipeline of the most promising projects and move them towards the clinic where they can be tested in people.

A team at UCLA was awarded $1.7 million to develop a way to genetically modify blood stem cells to help make the immune system resistant to the AIDS virus as a potential treatment and even cure for HIV/AIDS.

The 13 projects funded under CIRM's Discovery Quest Award program are listed here:

| APPLICATION | TITLE | INSTITUTION | CIRM COMMITTED FUNDING |
|---|---|---|---|
| DISC2-10591 | Preclinical development of an immune evasive islet cell replacement therapy for type 1 diabetes | ViaCyte | $1,470,987 |
| DISC2-10524 | Genome Editing of Sinusoidal Endothelial Stem Cells for Permanent Correction of Hemophilia A | City of Hope | $2,182,193 |

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| DISC2-10679 | Towards hepatocyte cell replacement therapy: developing a renewable source of human hepatocytes from pluripotent stem cells | Stanford | $2,201,136 |
| DISC2-10748 | Engineering Lifelong Cellular Immunity to HIV | U.C. Los Angeles | $1,701,178 |
| DISC2-10714 | iPS Glial Therapy for White Matter Stroke and Vascular Dementia | U.C. Los Angeles | $2,096,095 |
| DISC2-10604 | Stimulating endogenous muscle stem cells to counter muscle atrophy | Stanford | $2,198,687 |
| DISC2-10753 | Generation and in vitro profiling of neural stem cell lines to predict in vivo efficacy for chronic cervical spinal cord injury. | U.C. Irvine | $1,575,613 |
| DISC2-10751 | Silicon Nanopore Membrane encapsulated enriched-Beta Clusters for Type 1 Diabetes treatment | U.C. San Francisco | $1,113,000 |
| DISC2-10695 | Identification and Generation of Long Term Repopulating Human Muscle Stem Cells from Human Pluripotent Stem Cells | U.C. Los Angeles | $2,184,000 |
| DISC2-10747 | Targeting Cancer Stem Cells in Hematologic Malignancies | U.C. San Diego | $2,167,200 |
| DISC2-10559 | Development of immune invisible beta cells as a cell therapy for type 1 diabetes through genetic modification of hESCs | U.C. San Diego | $2,167,200 |
| DISC2-10525 | Development of a cellular therapeutic for treatment of epilepsy | Neurona Therapeutics | $1,616,536 |
| DISC2-10665 | Neural Stem Cell Relays for Severe Spinal Cord Injury | U.C. San Diego | $2,100,581 |

**About CIRM**

At CIRM, we never forget that we were created by the people of California to accelerate stem cell treatments to patients with unmet medical needs, and act with a sense of urgency to succeed in that mission.

To meet this challenge, our team of highly trained and experienced professionals actively partners with both academia and industry in a hands-on, entrepreneurial environment to fast track the development of today's most promising stem cell technologies.

With $3 billion in funding and approximately 300 active stem cell programs in our portfolio, CIRM is the world's largest institution dedicated to helping people by bringing the future of cellular medicine closer to reality.

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